Primary Sclerosing Cholangitis in Children

Status: RECRUITING

Eligibility Criteria

Sex: ALL
Age: 2 years to 25 years old
Healthy Volunteers: This study is NOT accepting healthy volunteers
Inclusion Criteria:

Patients with the clinical diagnosis of large or small duct PSC made at any time prior to enrollment are screened for eligibility to participate in this prospective cohort study. The site PI will determine eligibility following review of MRCP or ERCP images with the site radiologist to confirm presence of an abnormal cholangiogram at the time of diagnosis of large duct PSC. Liver histopathology obtained at the time of diagnosis of small duct PSC will be reviewed with the site pathologist prior to enrollment.

Individuals must meet all of the Inclusion criteria in order to be eligible to participate in the study:

1. Aged 2 through 25 years at time of screening.
2. Diagnosis of large duct PSC based on review of cholangiogram by MRC, ERC, or intraoperative cholangiogram (IOC) by the site radiologist and interpreted to be consistent with PSC, based on one or more of the following:

- Focal structuring of the bile duct(s)
- Dominant stricture of the common bile duct
- Saccular dilatation of bile duct(s)
- Beaded appearance of bile duct(s)
- Pruning appearance of the distal bile duct branches

AND/OR
3. Diagnosis of small duct PSC based on review of liver histopathology by the site pathologist and interpreted to be compatible with PSC:

- Probable small duct PSC: biopsy with ≥3 of 5 criteria: periductal edema, concentric inflammation, bile duct injury, ductular reaction, and neutrophils in bile ducts (cholangitis) OR...
- Definitive small duct PSC: Periductal fibrosis/ "onion skinning" around interlobular bile ducts or smaller profiles
    1. Stated willingness to comply with all study procedures and availability for the duration of the study.
    2. Able to provide informed consent/assent

Participants for the imaging study are eligible if they are:

1. Aged 8 through 25 years at the time of screening
2. No absolute contraindication to MRI
3. No skin condition that could be aggravated by MREL
4. Meet all other eligibility criteria of the PSC Observational Study
5. For whom none of the exclusion criteria apply

Exclusion Criteria:

An individual who meets any of the following criteria at baseline will be excluded from participation in this study.

1. History of liver transplantation
2. History bone marrow transplantation
3. History of primary or acquired immunodeficiency predisposing to secondary sclerosing cholangitis, for instance: hyper-IgM syndrome, severe combined immunodeficiency (SCID) syndrome, common variable immunodeficiency (CVID) syndrome, cartilage hair hypoplasia syndrome, or HIV/AIDS
4. History of histiocytosis, including Langerhans cell histiocytosis (LCH), or hemophagocytic lymphohistiocytosis (HLH)
5. History of ischemic cholangitis
6. History of portal vein thrombosis with biliopathy, veno-occlusive disease, or abdominal radiation vasculopathy
7. History of recurrent pyogenic cholangitis
8. History of biliary tract surgery for cholecystolithiasis prior to cholangiogram/liver biopsy evaluated to determine enrollment
9. History of biliary tract surgery for choledochal cyst
10. History of hepatocellular carcinoma, or hepatoblastoma
11. History of surgical biliary trauma
12. History of congenital cytomegalovirus (CMV) hepatitis
13. History of Sickle Cell Disease
14. History of cystic fibrosis, biliary atresia, Caroli disease/congenital hepatic fibrosis, or progressive familial intrahepatic cholestasis type 3/MDR3 disease
15. History of cardiac hepatopathy.
16. History of metabolic disorders, including Wilson's disease, glycogen storage disorder, Alpha-1 Antitrypsin deficiency
17. Diagnosis of systemic lupus erythematosus (SLE)
18. Concurrent pregnancy at the time of enrollment -

Conditions & Interventions

Conditions:
Primary Sclerosing Cholangitis, Liver Diseases, Cholangitis, Sclerosing
Keywords:
Pediatric Liver Disease, Primary Sclerosing Cholangitis

More Information

Description:
Primary sclerosing cholangitis (PSC) is a rare liver disease that damages the liver's bile ducts. Bile ducts are tiny tubes that carry bile from the liver to the small intestine. Bile is a liquid produced by the liver that helps us absorb and use the nutrients in the food we eat. In people with PSC, the bile backs up into the liver and will damage it, causing scarring of the liver. The purposes of this study are to: * Collect medical and other data to learn more about PSC, how it progresses, and identify factors that may cause the disease to progress more quickly. * Ask questions about how PSC symptoms affect your child's life to learn more about its impact on your child's daily functioning * Children with PSC who are seen at one of the participating clinical sites in the Childhood Liver Disease Research Network (ChiLDReN) will be asked to contribute information, DNA, and other specimens. The information and specimens will be available to investigators to carry out approved research aimed at learning more about the possible causes and long-term effects of PSC.

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